Systematic searches in the electronic databases MEDLINE, EMBASE and The Cochrane library were conducted and 53 online databases (including HTA agency websites, international ministries of health, and clinical trials. gov) were hand searched for clinical guidelines in the treatment of MO caused by RVO. **RESULTS:** Fifteen documents on treatment pathways or guidance used internationally were identified from the hand searches. No papers or abstracts were found from the electronic database searches. There were considerable between-jurisdiction differences in the guidance for the management of MO caused by RVO. These differences were consolidated to produce two amalgamated treatment pathways. In total, eight treatment positions for interventions in the treatment of RVO subtypes were identified. For one of the identified positions – treatment of ischaemic branch RVO – no licensed treatment currently exists. **CONCLUSIONS:** The described systematic methodology for the construction of treatment pathways may be used by manufacturers in early drug development decisions to identify unmet clinical needs, understand which treatment positioning may provide the most value, and identify future treatment comparators in the same indication. Guidelines to inform such commercial strategies may not be identifiable from electronic database searches alone with extensive hand searches being a necessity. Between jurisdiction guideline nuances also need to be taken into account when considering the target market for an intervention in development.

PSS59

OPHTHALMOLOGY: THERAPY TRENDS IN EUROPE BASED ON CLINICAL TRIAL REGISTRY DATA

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OBJECTIVES: Ophthalmology pharmaceutical market is growing worldwide due to rising aging population, new delivery technologies and changing lifestyle. However, challenges like patent expiry of major brands and lack of awareness still persists. Therefore, it is important to be aware of the upcoming treatment options, changing patients' needs and requirement of cost effective therapies. This analysis provides an overview of the recent trends and future scenario in Ophthalmology market. **METHODS:** Pharmaceutical companies sponsored clinical trials initiated from January 2011 to April 2014 in Glaucoma, Age-related Macular Degeneration (AMD), Diabetic Retinopathy (DR) / Diabetic Macular Edema (DME), Dry eye syndrome (DES) and Retinal vein occlusion (RVO) have been considered. Only Phase I - III trials listed on public registries have been considered. **RESULTS:** The data showed that >30% of the trials are being conducted on Glaucoma in USA, Europe, Asia and Australia. This is followed by AMD (24%), DR/DME (21%), DES (17%) and RVO (7%). Also, >50% ophthalmological trials are being conducted in USA; followed by Europe (~25%) and Asia (~20%). In Europe, 71 trials have been conducted on 48 molecules, of which 69% are chemical entities, 19% are biologicals and >10% are entities like RNAi (oligonucleotide, aptamers), DARPin. Eye drops (46%) and intravitreal injections (37%) are the key topical and parenteral formulations, respectively. 10% of the trials have been conducted on oral formulations. In Europe, EU5 countries comprise of 43% of the trials and Germany has maximum 37 trials. Novartis has conducted trials in maximum 30 countries, followed by Santen (19), Pfizer (15) and Allergan (13) in Europe. **CONCLUSIONS:** Based on the analysis, currently, Glaucoma, AMD and DR/DME are the major focus of the companies in ophthalmology. Though, biologicals and RNAi are being tested routinely, chemical entities are foremost modalities. Similarly, eye drops remain as preferred method of delivery with respect to other newer delivery techniques.

RESEARCH POSTER PRESENTATIONS - SESSION V

DISEASE-SPECIFIC STUDIES
CANCER – Clinical Outcomes Studies

PCN1

TREATMENT PATTERNS AND HEALTH OUTCOMES AMONG PATIENTS WITH RADIOIODINE-REFRACTORY DIFFERENTIATED THYROID CANCER IN THE UNITED STATES AND WESTERN EUROPE

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OBJECTIVES: Most patients with differentiated thyroid cancer (DTC) have an excellent prognosis after receiving standard treatment, consisting of surgery and often adjuvant radioactive iodine (RAI). However, a subgroup of patients prove to have progressive DTC which is refractory to RAI (RRDTC). Treatment options for RRDTC are limited. This study investigated the treatment patterns and health care resource utilization of patients with RRDTC. **METHODS:** Data were collected by performing a retrospective chart review study in the US and 5EU (France, Germany, Italy, Spain, UK) with physicians recruited from an online panel. Physicians provided clinical information on 1 to 4 of their RRDTC patients in an online survey. Demographics, disease history, treatment information, and health care resource were included and reported descriptively. Health care resource use was compared across treatment classes using general linear models. **RESULTS:** 231 physicians participated and provided a total of 700 patient charts (44.1% of charts were from the US and 11-12% from each 5EU country). 45.0% of patients were male with a mean age at diagnosis of 55.1 years [SD=12.4]. 52.0% of patients were treated with systemic treatment (e.g., 16.9% tyrosine kinase inhibitors [TKIs] only; 13.3% chemotherapy only). The remaining 48.0% were either in a watch and wait ("WW") period (20.1%) or were managed with non-systemic palliative therapies (27.9%; eg, external beam radiation). Overall, patients averaged 15.87 days hospitalized per year (due to disease related complications or side effects). Although not statistically significant (p>.05), a trend toward more days hospitalized from disease-associated complications was observed for patients managed with WW (Mean=9.21, respectively) and non-systemic treatment (Mean=8.27) than patients treated with chemotherapy (Mean=7.25) or TKIs (Mean=8.22). **CONCLUSIONS:** Among patients diagnosed with RRDTC, watch and wait and non-systemic treatment options remain common. A large direct cost burden may be observed given the frequent and long hospital stays.

PCN2

APPROVING DRUGS BASED ON EARLY STAGE DATA - HOW PHASE II TRIAL DATA CORRELATES WITH PHASE III OUTCOMES. CASE STUDY: NSCLC

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OBJECTIVES: There is increasing pressure on regulators from patients, physicians and industry for earlier access to pharmaceuticals for serious diseases. In reaction, in March 2014 the European Medicines Agency (EMA) announced it was piloting adaptive licensing, and the Medicines and Health care products Regulatory Agency (MHRA) unveiled their Early Access to Medicines Scheme. Nevertheless, there are questions over how, and if, Phase II trial benefits can be predictive of clinical advantages in Phase III studies, which this research aims to address. **METHODS:** Phase III data of any Non-Small Cell Lung Cancer (NSCLC) oncologic appraised by the EMA, or that had failed Phase III clinical trials, since 2002 was extracted along with its corresponding Phase II data. Statistical tests were conducted using Pearson's coefficient correlation. **RESULTS:** 12 oncologics were identified with both Phase II and III readouts, 6 of which met their Phase III trial primary endpoint. Overall Response Rates (ORRs) reported in Phase II trials varied from 0%-61% (mean 24%). 4/4 (100%) drugs with Phase II ORRs >30% met their primary endpoint vs. only 2/8 (25%) with ORRs ≤30%. Phase II ORRs were strongly correlated with Phase III Progression-Free Survival (PFS) (r^2=0.864, p<0.0005) and Overall Survival (OS) outcomes (r^2=0.858, p<0.001). Nevertheless, 5/6 drugs that failed their Phase III primary endpoints had comparative Phase II data indicating benefits versus these same comparators, most notably onartuzumab, whose Phase III trial was terminated early due to lack of efficacy, despite demonstrating significant OS benefits of 8.8 months in Phase II. **CONCLUSIONS:** In NSCLC, Phase II ORRs can be strongly predictive of the magnitude of PFS and OS readouts in Phase III trials. However, comparative advantages in Phase II trials seem to be poorly predictive of OS benefits in Phase III studies, raising questions over the appropriateness of approving drugs on early stage comparative data.

PCN4

CERVICAL HUMAN PAPILLOMA VIRUS (HPV) DNA PRIMARY SCREENING TEST RESULTS OF THE EXPERIENCE OF A REGIONAL LABORATORY IN CENTRAL ITALY

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OBJECTIVES: To investigate feasibility and effectiveness of a cervical screening program with DNA tests as preliminary assay versus usual cytology protocols in Umbria Region. **METHODS:** A large cohort of 35-64 aged women afferent to the unique regional laboratory was considered. The usual algorithm with cervical cytology as primary test was followed in January 2008–June 2010, whereas in August 2010–October 2011 high-risk human papillomavirus (HR-HPV) DNA test was used as primary screening. The cohorts were compared in terms of acceptance rate of invitation, cytological results, molecular results including HPV genotype, detection rate of histological lesions. **RESULTS:** A total of 31,228 women were invited: 21,249 were suggested to undergo classical cervical cytology screening, 9,979 HR-HPV DNA test as primary screening. A similar rate of adhesion (56.6% vs. 56.5%) was observed. Age-related differences were evidenced, with younger women (35-49) more prone to accept the invitation to HR-HPV DNA testing rather than usual cytology screening (61.6% vs. 55.5%; p<0.0001); analogously, uninvited younger women spontaneously requesting cervical screening were more prone to specifically request molecular than classical cytological testing (24.8% vs. 10.8%; p<0.0001). Among the 6,272 HR-HPV DNA testing women, 396 (6.4%) were positive, and, among them, 141 (36%) featured an altered cytology. All patients with altered cytology were suggested to undergo colposcopy and 106 out of 141 (75.1%) answered to the invitation. Among them, 89 (84%) featured abnormal histology with 48 (45.3%) CIN1 and 41 (38.7%) CIN2. If comparing the CIN2 detection rate within the two studied periods, it was almost doubled using the HR-HPV DNA than pap test as primary assay (0.64% vs. 0.37%; p=0.005). Finally, the implementation of the DNA test screening program did not increase total costs. **CONCLUSIONS:** Although with some limits, the introduction of HR-HPV DNA primary testing resulted feasible and effective, significantly increasing detection of severe lesions.

PCN5

COMPARATIVE EFFECTIVENESS OF TREATMENTS FOR RELAPSED OR REFRACTORY MANTLE CELL LYMPHOMA (R/R MCL), USING MATCHING ADJUSTED INDIRECT COMPARISON

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OBJECTIVES: Prognosis for relapsed or refractory (R/R) MCL patients with existing treatments is poor; most patients progress within ~4 months. Ibrutinib, an oral once daily Bruton's tyrosine kinase inhibitor showed durable single agent activity with good response rate in 111 R/R MCL patients and a median progression free survival (PFS) of 13.9 months. Ibrutinib received breakthrough designation and United States Food and Drugs Administration approval for use in MCL patients who received at least one prior therapy (R/R MCL). This indirect analysis aims to compare the efficacy

of ibrutinib to available treatments for R/R MCL patients. **METHODS:** A systematic literature review was conducted to identify clinical trials containing treatments of R/R MCL. Matching adjusted indirect comparison (MAIC), described by Signorovitch et al 2012, was utilized to obtain indirect relative treatment effect for ibrutinib compared to other treatments. Using individual patient level data (IPD), baseline characteristics of the ibrutinib trial patients were matched with the patients in the published studies to obtain overall response (ORR) and complete response (CR) rates based on balanced population between the ibrutinib and published studies. Kaplan Meir curves for overall survival and PFS of comparators were plotted alongside those of the matched ibrutinib patients. **RESULTS:** Nineteen studies evaluating various treatments were identified. Five trials evaluating bortezomib, BR (bendamustine, rituximab), FCM (fludarabine, cyclophosphamide, mitoxantrone), FCM-R (fludarabine, cyclophosphamide, mitoxantrone, rituximab), and rituximab-hyper-CVAD were considered for matching. Complete matching of the IPD was possible for the bortezomib, FCM and FCM-R studies. Ibrutinib showed statistically significant better odds of achieving ORR compared to bortezomib (OR 3.62; 95% CI 1.18-11.14) and FCM (OR 3.22; 95% CI 1.01-10.26). **CONCLUSIONS:** The indirect analysis suggests a potential for improved ORR compared to a few relevant treatments in patients with R/R MCL. Phase III comparative confirmatory data with ibrutinib are anticipated in late 2014.

PCN6

OVERALL SURVIVAL IN PATIENTS WITH HER2+ EARLY STAGE BREAST CANCER PATIENTS TREATED WITH TRASTUZUMAB IN THE US DEPARTMENT OF DEFENSE PRACTICE SETTING

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OBJECTIVES: The NSABP/NCCTG trial (Romond et al. NEJM 2005; 353: 1673-1684) established the efficacy of trastuzumab in the adjuvant treatment of HER2+ early stage breast cancer (HER2+BC). Yet, little is known about the patterns of use and outcomes of adjuvant trastuzumab in clinical practice. The study aimed to estimate the overall survival (OS) and relapse-free survival (RFS) of HER2+BC patients treated with adjuvant trastuzumab in the US Department of Defense (DOD) practice setting. **METHODS:** Adult women initiating adjuvant trastuzumab within 1 year of BC surgery were identified in the DOD health claims database (01/2003-12/2012). An algorithm based on secondary neoplasm ICD9 codes and treatment gaps and initiations was used to identify relapses. OS and RFS unadjusted rates at 3 and 4 years after the initiation of the adjuvant trastuzumab treatment were estimated from Kaplan-Meier plots. **RESULTS:** The study sample included 3,188 women (median age 63 years), followed for a median of 3.3 years after the initiation of trastuzumab and treated continuously with trastuzumab for a median of 12 months. Of these 3,188 women, 13.8% received neo-adjuvant therapy prior to the surgery, 17.7% relapsed, and 7.9% died during the follow-up. The OS rates at 3 and 4 years were 93.2% (95% CI 92.1%-94.2%) and 90.0% (88.6%-91.2%), respectively. The corresponding RFS rates were 78.8% (77.1%-80.3%) and 75.8% (74.0%-77.5%), respectively. **CONCLUSIONS:** The findings suggest that most HER2+BC patients in the DOD practice setting received per-label trastuzumab treatment (for 52 weeks) and had OS rates that are similar to the OS rates that were previously observed in the NSABP/NCCTG clinical trial (90.0% vs. 93% at four years). The lower RFS rates observed in this study versus the NSABP/NCCTG trial (75.8% vs. 85.7% at 4 years), may be partially explained by differences in the characteristics of the patients, including age.

PCN7

THE RELATIVE EFFICACY OF TREATMENTS IN FIRST-LINE MANAGEMENT OF NEWLY DIAGNOSED CHRONIC MYELOID LEUKAEMIA: SYSTEMATIC LITERATURE REVIEW AND INDIRECT COMPARISON

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OBJECTIVES: To assess the relative efficacy of first-line treatments in chronic myeloid leukaemia (CML), an updated systematic literature review (SLR) and indirect comparison (IC) were conducted with follow-up period up to 48 months. **METHODS:** We updated a SLR initially conducted in 2011. Medical databases were interrogated systematically in January 2014 to identify trials comparing first-line treatments for CML. Using a fixed-effect Bayesian model implemented in WinBUGS, ICs were made to calculate relative efficacy (cumulative complete cytogenetic response (CCyR) and major molecular response (MMR)) for dasatinib, nilotinib and imatinib. **RESULTS:** Nineteen randomised controlled trials (RCTs) were included in the SLR, 10 were eligible for inclusion in the IC. Compared with imatinib 400mg by 12 months, odds of cumulative CCyR were significantly greater for dasatinib 100mg [odds ratio (OR) 2.25,95% credible interval (CrI) 1.55-3.15], nilotinib 600mg [OR 2.23 (95% CrI 1.50-3.21)] and 800mg [OR 1.94 (95% CrI 1.31-2.78)]. By 24 months compared with imatinib 400mg, the odds remained significantly higher with nilotinib 600mg [OR 2.03 (95% CrI 1.28-3.10)] and 800mg [OR 1.70 (95% CrI 1.08-2.55)] and higher, but not significant with dasatinib 100mg [OR 1.41 (95% CrI 0.85-2.22)]. By 12,24,36 and 48 months respectively, compared with imatinib 400mg, the odds of a MMR were: dasatinib 100mg [OR 2.22 (95% CrI 1.52-3.15) / 2.09 (1.45-2.93) / 1.76 (1.22-2.48) / 1.90 (1.27-2.74)] nilotinib 600mg [OR 2.86 (95% CrI 1.95-4.08) / 3.24 (2.26-4.54) / 2.45 (1.70-3.43) / 2.54 (1.75-3.59)] and 800mg [OR 2.76 (95% CrI 1.88-3.94) / 2.60 (1.82-3.62) / 2.12 (1.48-2.95)/ 2.18 (1.51-3.06)]. For both outcomes at all time points, there was no significant difference between dasatinib and nilotinib. **CONCLUSIONS:** Analysis including all available RCTs suggests that second-generation tyrosine kinase inhibitors dasatinib and nilotinib are more efficacious than imatinib 400mg and should be treatments of choice in newly diagnosed CML.

PCN8

TREATMENTS FOR EGFR MUTATION-POSITIVE (M+) NSCLC PATIENTS – A NETWORK META-ANALYSIS (NMA) BY MUTATION TYPE

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OBJECTIVES: Lung cancer is one of the most common causes of cancer-related deaths world-wide. Afatinib is an irreversible ErbB family blocker showing superior efficacy in EGFRm+ NSCLC as 1st-line treatment compared to standard-of-care chemotherapy. To date, no head-to-head trial results exist to compare afatinib, gefitinib or erlotinib. Previous NMAs have compared the three treatments on an ITT level, but not by mutation type. This analysis attempts to fill this gap. **METHODS:** Based on the EGFRm+ study network requested by NICE a Bayesian approach NMA was conducted to estimate relative treatment effects of afatinib versus erlotinib and gefitinib for progression free survival (PFS) and overall survival (OS) per mutation type (common mutations: Del19 and L858R). **RESULTS:** 9 studies were included, 8 reported PFS and 5 OS by mutation type, respectively. Results from fixed effects models are reported. Afatinib significantly improved PFS in common mutations versus gefitinib (HR 0.43: CrI 0.24; 0.75) and erlotinib (HR 0.60; CrI 0.39; 0.91). Results also favored afatinib for both EGFR mutation subgroups Del19 and L858R, but did not reach statistical significance. Afatinib showed a high probability (>80%) of being the best treatment both for common mutations and per mutation type. For OS, a trend favoring afatinib was shown in particular for Del19. The probability of afatinib being best for Del19 was >70%. For L858R no difference in OS was detected between the TKIs. **CONCLUSIONS:** In line with findings from previous NMAs, this analysis by mutation type confirms both for PFS and OS a consistent trend towards superiority of afatinib versus reversible TKIs. Afatinib appears to be the best treatment option for patients with common mutations, in particular Del19 mutations. A direct trial-based comparison of the efficacy of these agents is warranted to clarify their relative benefits.

PCN9

HEALTH CARE COSTS IN PATIENTS TREATED WITH IPILIMUMAB FOR ADVANCED MELANOMA RESULTS OF A RETROSPECTIVE CHART REVIEW

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OBJECTIVES: This analysis described health care costs over time—excluding ipilimumab drug costs—stratified by survival duration and baseline ECOG status, in patients receiving ipilimumab for advanced melanoma in the US community setting. **METHODS:** We analyzed data from a retrospective chart review of patients with unresectable stage III/IV melanoma treated with ipilimumab as first-line monotherapy between 04/2011 and 09/2012. Hospitalizations, emergency department visits, subsequent chemotherapy, radiation, surgeries, nursing home, and hospice visits costs were estimated using published sources and tariffs. Total costs, excluding ipilimumab drug costs, were calculated for 3 periods: treatment regimen (between first and last ipilimumab doses); post-regimen; and pre-death (within 90 days of death). Monthly costs were compared for the total population and stratified by baseline ECOG status (0 vs. ≥1, when available) and survival (<1 year vs. ≥1 year) using Wilcoxon rank sum tests. **RESULTS:** Data were abstracted from 273 patient charts at 34 sites. Excluding ipilimumab drug costs, total monthly costs during the treatment regimen, post-regimen, and pre-death periods were $690, $2151, and $5123, respectively. Total monthly costs across all study periods were higher for patients with ECOG ≥1 (n=135) vs. ECOG=0 (n=104) (p=0.0294), particularly in the pre-death period ($5987 vs. $3460, respectively; p=0.0143). A similar pattern was observed for patients surviving <1 year (n=109) vs. ≥1 year (n=122) (p<0.0001), with a difference of $9524 vs. $2955 (p<0.0001) during the pre-death period (42 patients still alive after <1 year follow-up were excluded from this analysis). Key cost drivers were hospitalizations (32.4% of total costs), followed by non-ipilimumab chemotherapy (23.1%), hospice care (19.1%), and nursing home stays (12.5%). **CONCLUSIONS:** In this population, monthly costs were significantly lower during the treatment regimen period than in subsequent periods. Survival ≥1 year and baseline ECOG=0 were associated with significantly lower total monthly costs, particularly in the pre-death period.

PCN10

SYSTEMATIC REVIEW OF RELAPSED OR REFRACTORY MANTLE CELL LYMPHOMA (MCL) CLINICAL TRIALS: IMPLICATIONS FOR DECISION MODELING

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OBJECTIVES: No standard of care exists for patients with relapsed or refractory mantle cell lymphoma (MCL) and treatment options are limited. This study sought to synthesize the clinical evidence of current treatments for relapsed or refractory MCL, its limitations, and discuss the implications for decision making. **METHODS:** A systematic literature review was conducted in MEDLINE of phase II, III, or IV clinical trials published in English between January 1, 2001 and May 2, 2013. Supplemental searches included EHA, AACR, ASCO, and ASH 2011–2013 conference proceedings. **RESULTS:** Results of the review indicate a paucity of evidence relevant for decision making. Of 808 records reviewed, 17 trials in the R/R MCL population were identified, only three of which were randomized controlled trials; all others were single-arm trials. For most treatments, only one trial was available. These factors made it infeasible to conduct a meta-analysis or indirect comparison. Furthermore, there was a large amount of heterogeneity in the patient populations, trial designs, and reported outcomes, making it difficult to compare outcomes across trials. Finally, of the 17 trials identified, five reported progression-free survival (PFS) Kaplan–Meier (K-M) graphs and only three reported overall survival (OS)